6 January 2017
EMA/758032/2016
Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Pegylated recombinant human interleukin-10 for the treatment of pancreatic cancer

On 12 December 2016, orphan designation (EU/3/16/1804) was granted by the European Commission to Larode Ltd, United Kingdom, for pegylated recombinant human interleukin-10 (also known as AM0010) for the treatment of pancreatic cancer.

What is pancreatic cancer?

Pancreatic cancer is cancer of the pancreas, a small organ that lies behind the stomach. The pancreas has two functions: to produce a fluid that helps with the digestion of food, and to produce hormones such as insulin. Due to the absence of symptoms in the early stages of pancreatic cancer, the majority of patients are diagnosed when the cancer has spread nearby or to other parts of the body.

Pancreatic cancer is a very severe and life-threatening disease that is associated with shortened life expectancy.

What is the estimated number of patients affected by the condition?

At the time of designation, pancreatic cancer affected approximately 2.4 in 10,000 people in the European Union (EU). This was equivalent to a total of around 123,000 people*, and is below the ceiling for orphan designation, which is 5 people in 10,000. This is based on the information provided by the sponsor and the knowledge of the Committee for Orphan Medicinal Products (COMP).

What treatments are available?

At the time of designation, several medicines were authorised in the EU for treating pancreatic cancer. The choice of treatment depended on several factors, including how far the disease had advanced. Treatments included surgery, radiotherapy (treatment with radiation) and chemotherapy (medicines to treat cancer).

*Disclaimer: For the purpose of the designation, the number of patients affected by the condition is estimated and assessed on the basis of data from the European Union (EU 28), Norway, Iceland and Liechtenstein. This represents a population of 513,700,000 (Eurostat 2016).

30 Churchill Place ● Canary Wharf ● London E14 5EU ● United Kingdom
Telephone +44 (0)20 3660 6000 **Facsimile** +44 (0)20 3660 5555
Send a question via our website www.ema.europa.eu/contact

The sponsor has provided sufficient information to show that this medicine might be of significant benefit for patients with pancreatic cancer. Early data indicate that it can be used in combination treatments for advanced cancer that has worsened despite treatment with gemcitabine (a cancer medicine). In addition, the improvement seen so far with this medicine compares well with that seen with other treatments. These assumptions will need to be confirmed at the time of marketing authorisation in order to maintain the orphan status.

How is this medicine expected to work?

This medicine is a type of immunotherapy, which means that it acts on the body's immune system (the body's natural defences). It activates white blood cells known as CD8+ T cells. These cells can infiltrate pancreatic cancer tumours, where they are expected to kill cancer cells and improve survival.

What is the stage of development of this medicine?

The effects of the medicine have been evaluated in experimental models.

At the time of submission of the application for orphan designation, clinical trials with the medicine in patients with pancreatic cancer were ongoing.

At the time of submission, the medicine was not authorised anywhere in the EU for pancreatic cancer. Orphan designation had been granted in the United States for the condition.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 4 November 2016 recommending the granting of this designation.

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Opinions on orphan medicinal product designations are based on the following three criteria:

- the seriousness of the condition;
- the existence of alternative methods of diagnosis, prevention or treatment;
- either the rarity of the condition (affecting not more than 5 in 10,000 people in the EU) or insufficient returns on investment.

Designated orphan medicinal products are products that are still under investigation and are considered for orphan designation on the basis of potential activity. An orphan designation is not a marketing authorisation. As a consequence, demonstration of quality, safety and efficacy is necessary before a product can be granted a marketing authorisation.

For more information

Sponsor's contact details:

Contact details of the current sponsor for this orphan designation can be found on EMA website, on the medicine's rare disease designations page.

For contact details of patients' organisations whose activities are targeted at rare diseases see:

- Orphanet, a database containing information on rare diseases, which includes a directory of patients' organisations registered in Europe;
- European Organisation for Rare Diseases (EURORDIS), a non-governmental alliance of patient organisations and individuals active in the field of rare diseases.

Translations of the active ingredient and indication in all official EU languages[1], Norwegian and Icelandic

Language	Active ingredient	Indication
English	Pegylated recombinant human interleukin-10	Treatment of pancreatic cancer
Bulgarian	Пегилиран рекомбинантен човешки интерлевкин-10	Лечение на рак на панкреаса
Croatian	Pegilirani rekombinantni ljudski interleukin-10	Liječenje raka gušterače
Czech	Pegylovaný rekombinantní lidský interleukin - 10	Léčba karcinomu pankreatu
Danish	Pegyleret rekombinant human interleukin-10	Behandling af pancreascancer
Dutch	Gepegyleerd recombinant humaan interleukine -10	Behandeling van pancreaskanker
Estonian	Pegüleeritud rekombinantne inimese interleukiin-10	Pankreasevähi ravi
Finnish	Pegyloitu rekombinantti ihmisen interleukeeni 10	Haimasyövän hoito
French	Interleukine 10 recombinante humaine **pégylée**	Traitement du cancer pancréatique
German	Pegyliertes rekombinantes Interleukin-10	Behandlung des Pankreaskarzinoms
Greek	Πεγκυλιωμένη ανασυνδυασμένη ανθρώπινη ιντερλευκίνη-10	Θεραπεία καρκίνου του παγκρέατος
Hungarian	Pegylált rekombináns humán interleukin-10	Hasnyálmirigyrák kezelése
Italian	Interleuchina 10 umana recombinante pegilata	Trattamento del cancro pancreatico
Latvian	Pegilēts rekombinants cilvēka interleikīns-10	Aizkuņģa dziedzera vēža ārstēšana
Lithuanian	Pegiliuotas rekombinantinis žmogaus interleukinas-10	Kasos vėžio gydymas
Maltese	Peġilat rikombinanti uman interleukin-10	Kura tal-kanċer tal-frixa
Polish	Pegylowana rekombinowana ludzka interleukina 10	Leczenie raka trzustki
Portuguese	Interleucina-10 humana recombinante peguilada	Tratamento do carcinoma do pâncreas
Romanian	Interleukină-10 pegilată recombinantă umană	Tratamentul cancerului pancreatic
Slovak	Pegylovaný rekombinantný ľudský interleukín-10	Liečba rakoviny pankreasu
Slovenian	Pegiliran rekombinanten človeški interlevkin-10	Zdravljenje raka trebušne slinavke
Spanish	Interleucina-10 humana recombinante peguilada	Tratamiento del cáncer de páncreas
Swedish	männskiligt pegylerat rekombinant interleukin-10	Behandling av pankreascancer
Norwegian	Pegylert rekombinant humant interleukin-10	Behandling av pankreascancer
Icelandic	Pegýlerað raðbrigða manna interleukin-10	Meðferð briskrabbameins

[1] At the time of designation